REGENXBIO Announces Agreement to Monetize Portion of Zolgensma® Royalties for $200 Million

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- Agreement with Healthcare Royalty enables REGENXBIO to advance its broad gene therapy pipeline, including pivotal program for RGX-314

ROCKVILLE, Md., Dec. 22, 2020 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that it has entered into an agreement to sell a portion of the royalty rights due to REGENXBIO from Novartis Gene Therapies from the net sales of Zolgensma® to entities managed by Healthcare Royalty Management, LLC (HCR) for a gross purchase price of $200 million. This transaction provides immediate, non-dilutive capital to REGENXBIO for continued innovation in the development of potential breakthrough gene therapies for patients and completion of its internal manufacturing capabilities.

"Our rapidly advancing internal pipeline has enabled us to broaden the potential impact that gene therapies can have for patients in both large and orphan indications. This agreement with HCR provides us with significant additional non-dilutive funding to continue our momentum in the clinic focused on RGX-314 and our rare neurodegenerative disease platform, including RGX-121, as well as the opportunity to develop new innovations for patients in other disease areas," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "The capital will continue to support our pipeline transition into late-stage development and the establishment of internal manufacturing facilities with 2,000 liter scale using our platform suspension cell culture process for emerging commercial requirements, so that we can continue to work towards our mission of improving the lives of patients."

Under the terms of the agreement, REGENXBIO will receive $200 million from HCR as an upfront payment in exchange for REGENXBIO's royalty rights from the net sales of Zolgensma, including a portion of the royalties received in the fourth quarter of 2020, up to 1.3 times the purchase price until November 7, 2024 or, if such cap is not met by November 7, 2024, up to 1.5 times the purchase price thereafter. If either cap is met, the royalty rights would revert to REGENXBIO.

Zolgensma is currently approved for the treatment of Spinal Muscular Atrophy (SMA) in the United States, Japan, Europe, Brazil and Canada. Novartis is also pursuing registration in additional countries.

"Zolgensma is a truly innovative treatment for SMA based on REGENXBIO's NAV technology which we believe demonstrates the transformational impact that gene therapy can offer patients. We are pleased to partner with REGENXBIO in this royalty agreement to recognize the value of this therapy, and to enable the further development of their internal pipeline of new gene therapies for patients in need," said Clarke B. Futch, Managing Partner & Chairman of HCR.

Morgan Stanley & Co. LLC served as sole structuring agent and Covington & Burling LLP served as counsel to REGENXBIO. Morgan Lewis & Bockius LLP acted as counsel to HCR.

About REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

About HCR

HCR is a private investment firm that purchases royalties and uses debt-like structures to invest in commercial or near-commercial stage biopharmaceutical assets. HCR has raised $5.7 billion in cumulative capital commitments with offices in Stamford (CT), San Francisco, Boston and London For more information, visit www.healthcareroyalty.com

Forward-Looking Statements

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's clinical trials, future operations and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's

Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2019, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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